

## (12) INTERNATIONAL APPLICATION PUBLISHED UNDER THE PATENT COOPERATION TREATY (PCT)

(19) World Intellectual Property Organization International Bureau



(43) International Publication Date  
28 October 2004 (28.10.2004)

PCT

(10) International Publication Number  
WO 2004/091548 A2

- (51) International Patent Classification<sup>7</sup>: A61K (81) Designated States (unless otherwise indicated, for every kind of national protection available): AE, AG, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BW, BY, BZ, CA, CH, CN, CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, MZ, NA, NI, NO, NZ, OM, PG, PH, PL, PT, RO, RU, SC, SD, SE, SG, SK, SL, SY, TJ, TM, TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, YU, ZA, ZM, ZW.
- (21) International Application Number: PCT/US2004/009289 (84) Designated States (unless otherwise indicated, for every kind of regional protection available): ARIPO (BW, GH, GM, KE, LS, MW, MZ, SD, SL, SZ, TZ, UG, ZM, ZW), Eurasian (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM), European (AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HU, IE, IT, LU, MC, NL, PL, PT, RO, SE, SI, SK, TR), OAPI (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG).
- (22) International Filing Date: 15 April 2004 (15.04.2004)
- (25) Filing Language: English
- (26) Publication Language: English
- (30) Priority Data: 60/462,895 15 April 2003 (15.04.2003) US
- (71) Applicant (for all designated States except US): AVALON PHARMACEUTICALS, INC [US/US]; 20358 Seneca Meadows Parkway, Germantown, MD 20876 (US).
- (72) Inventors; and
- (75) Inventors/Applicants (for US only): STROVEL, Jeffrey, W. [US/US]; 14622 Keeneland Circle, North Potomac, MD 20878 (US). CAIN, Colyn, B. [US/US]; 4309 Kentbury Drive, Bethesda, MD 20814 (US). HORRIGAN, Steven, K [US/US]; 1895 Millboro Drive, Potomac, MD 20854 (US). AUGUSTUS, Meena [US/US]; 3215 Hollyhock Drive, Burtonsville, MD 20866 (US).
- (74) Agents: GRANT, Alan, J. et al.; Carella, Byrne, Bain, Gilfillan, Cecchi, Stewart & Olstein, 5 Becker Farm Road, Roseland, NJ 07068 (US).

## Published:

- without international search report and to be republished upon receipt of that report
- with sequence listing part of description published separately in electronic form and available upon request from the International Bureau

For two-letter codes and other abbreviations, refer to the "Guidance Notes on Codes and Abbreviations" appearing at the beginning of each regular issue of the PCT Gazette.

WO 2004/091548 A2

(54) Title: DETERMINING CANCER-LINKED GENES AND THERAPEUTIC TARGETS USING MOLECULAR CYTOGENETIC METHODS

(57) **Abstract:** Methods for identifying potential therapeutic agents, such as anti-tumor agents, based on their modulation of the expression of specified genes, especially genes mapping to specific chromosomal regions, are disclosed. Also described are methods for diagnosing cancerous, or potentially cancerous, conditions as a result of the expression, or patterns of expression, of such genes, including detecting changes in levels of gene copy number and/or level of amplification of the said gene, or sets of genes, to detect and/or diagnose the cancer. Methods for detecting or determining functionally related genes, as well as methods for treating cancer based on targeting expression products of such genes, determining genes involved in the cancerous process and the success and/or response rates and survival statistics for cancer patients on treatment are encompassed by the invention. Also encompassed are methods involving determining the modulated expression of the genes in these regions of interest (ROIs) as pharmacodynamic/pharmacogenetic/surrogate markers and/or for patient profiling prior to accrual for clinical trials/treatments based on the identification of these genes as validated gene/drug targets in various cancer tissue types.